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Lidow Family Research Chair and Professor,
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EXPERIENCE	
1969 - 1970	Graduate Teaching Assistant in Microbiology, University of Connecticut, Storrs, CT, USA
1970 - 1971	Graduate Teaching Assistant in Genetics, University of Connecticut, Storrs, CT, USA
1971 - 1973	Military Service: Space Surveillance Officer, Ent Air Force Base, Colorado Springs, CO.,
	Honorable Discharge
1975 - 1975	Instructor in Genetics (summer session), University of Connecticut, Storrs, CT, USA
1977 - 1978	Teaching Assistant in Molecular Biology and Microbiology, Brown University, Providence,
	RI, USA
1976 - 1979	Postdoctoral Research Fellow under Dr. Arthur Landy, Div. of Biology and Medicine,
	Brown University, Providence, RI, USA
1980 - 1984	Assistant Research Scientist, Department of Molecular Genetics, City of Hope Research
4000 4001	Institute, Duarte, CA, USA
1985 - 1991	Teaching - UCLA Graduate Dept. of Microbiology, Los Angeles, CA, USA
1984 - 1989	Associate Research Scientist, Department of Molecular Genetics, Beckman Research Institute of City of Hope, Duarte, CA, USA
1988 - 1992	Associate Chairman, Division of Biology, Beckman Research Institute of City of Hope,
	Duarte, CA, USA
1988 - 1994	Adjunct Research Professor of Microbiology, Loma Linda School of Medicine, Loma Linda,
	CA, USA
1990 - 7/94	Research Scientist, Department of Molecular Genetics, Beckman Research Institute of
	City of Hope, Duarte, CA, USA
1990 - 7/94	Director, Department of Molecular Genetics, Beckman Research Institute of City of Hope, Duarte, CA, USA
1992 - 7/94	Chairman, Division of Biology, Beckman Research Institute of City of Hope, Duarte, CA, USA
8/94 - 3/96	Associate Director, Center for Molecular Biology and Gene Therapy, Loma Linda University, Loma Linda, CA, USA
10/94-Present	Adjunct Professor, Division of Biology and Department of Hematology, BMT, City of Hope National Medical Center, Duarte, CA, USA
3/96 - 7/96	Director, Center for Molecular Biology and Gene Therapy, Loma Linda University, Loma Linda, CA, USA
8/96-Present	Adjunct Professor, Department of Basic Sciences, Loma Linda University, Loma Linda, CA,
	USA
8/96 - 8/01	Director, Department of Molecular Biology, Section Head, Gene Regulation, Beckman Research Institute of City of Hope, Duarte, CA, USA
1/98-Present	Dean, Graduate School of Biological Sciences, City of Hope National Medical Center and
	Beckman Research Institute of City of Hope, Duarte, CA., USA
2001-Present	Associate Director for Basic Research, City of Hope Comprehensive Cancer Center, City of
	Hope National Medical Center, Duarte CA., USA
2001-Present	Associate Chair, Shared Resources Advisory Committee, City of Hope Comprehensive
	Cancer Center, City of Hope National Medical Center, Duarte CA., USA
2001-Present	Co-Program Director, Cancer Biology Program, City of Hope Comprehensive Cancer
Anna h	Center, City of Hope National Medical Center, Duarte CA., USA
2001-Present	Adjunct Prof., Div. of Biomedical Sciences, Univ. of California, Riverside, Riverside, CA

1996-Present Chair and Professor, Department of Molecular Biology, Beckman Research Institute of the

City of Hope, Duarte, CA

2008-Present Lidow Family Endowed Research Chair

#### **EDUCATION**

1969 B.A., Biology, University of New Hampshire, Durham NH, USA

1971 M.S., Microbial Genetics, University of Connecticut, Storrs, CT, USA (Dr. Claire Berg)
1976 Ph.D., Microbial Genetics, University of Connecticut, Storrs, CT, USA (Dr. Claire Berg)

1976 - 1979 Postdoctoral Fellow, Brown University, Providence, RI, USA (Dr. Arthur Landy)

## **HONORS AND AWARDS**

Phi Beta Kappa, Phi Sigma, Phi Kappa Phi and Sigma XI Honor Societies

U.S. Air Force Financial Assistance Grant

University of Connecticut State Scholarship

University of Connecticut Demi-Fellowship

University of Connecticut Summer Scholarship

NDEA Graduate Fellowship

National Science Foundation Predoctoral Fellowship

U. S. P. H. S. Postdoctoral Fellowship

Walter MacPherson Society Research Award, Loma Linda Univ. School of Medicine, 1990

American Association for Clinical Chemistry Outstanding Speaker Award, 1991

Gallery of Scientific and Medical Achievement, City of Hope National Medical Center, 1993

Merit Award, Division of AIDS, National Institute of Allergy and Infectious Diseases, 2002

Cozzarelli Award, National Academy of Sciences 2008

#### PROFESSIONAL MEMBERSHIPS

American Association for the Advancement of Science

American Society for Microbiology

American Society for Biochemistry and Molecular Biology

Genetics Society of America

American Society for Gene Therapy

**RNA Society** 

Oligonucleotide Therapeutics Society, Inc.-President

## **CURRENT EDITORIAL BOARDS**

2006- Present	Oligonucleotides, Co-Editor in Chief
1997 - Present	Frontiers in Bioscience, Editor
1998 - Present	Gene Therapy and Molecular Biology
1999 - Present	Molecular Therapy, Associate Editor
2002 - Present	Cancer Gene Therapy, Editorial Board
2005 - Present	Biotechnology Journal, Editorial Board
2005 - Present	Journal of Biological Chemistry, Editorial Board
2005 - Present	BioTechniques, Editorial Board
2006 - Present	Recent Patents in Biotechnology, Editorial Board
2007 - Present	Open Biotechnology, Editorial Board
2007 - Present	Open Genetics, Editorial Board
2008 - Present	Gene Therapy
2008 - Present	Silence, Editorial Board

# SCIENTIFIC CONSULTANT AND ADVISORY BOARDS

2000 - 2005

Scientific Consultant, Immusol Inc.

2003 - 2005	Scientific Advisory Board, International Therapeutics, Inc
2001 - 2006	Scientific Consultant, VIRxSYS
2003 - 2008	Board of Directors, CytoGenix, Inc.
2003 - 2005	Chairman, Scientific Advisory Board, Benitec Inc., Australia Ltd.
2007 - Present	Scientific Advisory Board, Benitec, Inc. Australia Ltd.
2004 - Present	Member, External Advisory Committee, Center of Biomedical Research Excellence
	(COBRE), Roger Williams Hospital, Providence, R.I.
2005 - Present	Advisory Board, American Society of Gene Therapy
2005 - Present	Advisory Board Genome Canada funded program at Sherbrooke University, Quebec
2006 - Present	Scientific Advisory Board, NanoViricides Inc.
2005 - 2007	Co-Founder, Calando Pharmaceuticals, Inc.
2007	Co-Founder and Scientific Advisory Board Chairman, Dicerna Pharmaceuticals Inc.

# **AD HOC GRANT REVIEWER**

1992 - 1994	Beckman Foundation
1992	University of North Carolina, Biotechnology Program
1991 - 1994	NSF
1991 -1997	Veteran's Administration
1988 - 1994	City of Hope Cancer Center Advisory Panel
1993	NIH National Cooperative Drug Discovery Group Program
1994	NIAID Special Antiviral Study Section
1994 - 1995	Medical Research Council of the United Kingdom
1996 - Present	Leukemia Foundation, United Kingdom
1996	AIDS and Related Research Study Section, NIAID, National Institutes of Health

# GRANT REVIEW PANELS AND TASK FORCE MEMBERSHIPS 1993 -1996 National Science Foundation - Biochemist

1993 - 1990	National Science Foundation - Biochemistry and Genetics
1991 - 1996	American Cancer Society - Postdoctoral Fellowship Committee
1997 - 2001	AIDS and Related Research Study Section, NIAID, National Institutes of Health
1999 - 2001	Chairman, AIDS and Related Research Study Section, NIAID, National Institutes of Health
2004 - Present	Chairman, Small Business Innovative Research Study Section, NIAID, National Institutes of Health
2000 - 2006	University wide Task Force on AIDS - University of California
2004 - 2006	Chairman University of California Wide Task Force on AIDS
2008 - Present	AIDS Discovery and Development of Therapeutics Study Section, National Institute of Health

#### PATENTS

Patent Number 4,874,853	Synthetic Oligonucleotides Useful in Diagnosis of Chronic Myelogenous Leukemia
Patent Number 5,110,802	Oligonucleotide Phosphonates and Method of Inhibiting a Human Immunodeficiency Virus <i>In Vitro</i> Utilizing said Oligonucleotide Phosphonates
Patent Number 5,272,262	Method for the Production of Catalytic RNA in Bacteria
Patent Number 5,750,380	DNA Polymerase Medicated Synthesis of Double Stranded Nucleic Acids
Patent Number 5,827,935	Chimeric tRNA <sup>LYS</sup> -Ribozyme Molecules
Patent Number 5,869,249	Method for Amplification and Detection of RNA and DNA Sequences
Patent Number 5,985,661	Anti-HIV Ribozymes
Patent Number 5,695,938	Anti-HIV Ribozymes
Patent Number 8,522,356	Inhibitors and Target Molecule Co-Localization
	7.

Patent Number 6,100,087 Ribozymes Targeted to Human CCR5 mRNA

Patent Number 5,144,019 Ribozyme Cleavage of HIV-1 RNA Ribozyme Cleavage of HIV RNA

Patent Number 5,149,796 Chimeric DNA-RNA Catalytic Sequences
Patent Number 6,096,715 Chimeric DNA-RNA Catalytic Sequences

Patent Number 6,379,931 Chimeric DNA/RNA Ribozymes Containing Propanediol Patent Number: 6,562,570 B1 Method for Identifying Accessible Binding Sites on RNA

Patent Number: 10,365,643 Methods for Producing Interfering RNA Molecules in Mammalian

Cells and Therapeutic Uses for Such Molecules

Patent Number: 5,783,391 Method for Amplification and Detection of RNA and DNA Sequences
Patent Number: 5,622,820 Method for Amplification and Detection of RNA and DNA Sequences

Patent Number: 699, 5258 B1 Nucleolar Targeting of Therapeutics Against HIV

## Mentored Ph.D. graduates

James Deshler, Microbiology, University of California, Los Angeles, CA, USA
Garrett Larson, Microbiology, University of California, Los Angeles, CA, USA
Pairoj Chang, Biochemistry, Loma Linda University, Loma Linda, CA, USA
Nerida Taylor, Physiology, Loma Linda University, Loma Linda, CA, USA
David Elkins, Microbiology and Molecular Genetics, Loma Linda University, Loma Linda, CA, USA
Juan Lin, Microbiology and Molecular Genetics, Loma Linda University, Loma Linda, CA, USA
John Francis, Microbiology and Molecular Genetics, Loma Linda University, Loma Linda, CA, USA
Zongli Chang, Microbiology and Molecular Genetics, Loma Linda University, Loma Linda, CA, USA
Yuksel Yildez, Microbiology and Molecular Genetics, Loma Linda University, Loma Linda, CA, USA
Ali Eshani, Molecular Biology, Beckman Research Institute of the City of Hope, CA, USA
Shou Gu, Molecular Biology, Beckman Research Institute of the City of Hope, CA, USA
Bret Heale, Molecular Biology, Beckman Research Institute of the City of Hope, CA, USA

#### **PUBLICATIONS**

Rossi, J.J. and Berg, C.M. (1971) Differential recovery of auxotrophs after penicillin enrichment in Escherichia coli. J. Bacteriol. 106: 297-300.

Berg, C.M. and Rossi, J.J. (1974) Proline excretion and indirect suppression in Escherichia coli and Salmonella typhimurium. J. Bacteriol, 118: 928.

Berg, C.M., Rossi, J.J., Coleman, J.V. and Conlon, M. (1975) Procedure for isolating mutants defective in metabolite transport or utilization. *J. Bacteriol.* **121**: 1216-1218.

Rossi, J.J. and Berg, C.M. (1975) The effect of enrichment procedure upon auxotroph recovery in *Escherichia coli* K-12. *Antimicrob. Agents and Chemotherapy* 7: 110-112.

Anderson, S., Berg, C.M. and Rossi, J.J. (1975) An inexpensive membrane filtration manifold. *Analytical Biochem.* **69**: 655-665.

Rossi, J.J., Vender, J., Berg, C.M. and Coleman, W.H. (1977) Partial purification and some properties of Æ-1-pyrroline-5-carboxylate reductase from Escherichia coli. J. Bacteriol. 129: 108-114.

Rossi, J.J., Vender, J. and Berg, C.M. (1977) Proline excretion in Escherichia coli: A comparison of an argD+ strain and a proline-excreting argD- derivative. Biochem. Genetics 15: 287-296.

Rossi, J.J., Ross, W., Egan, J., Lipman, D.J. and Landy, A. (1979) Structural organization of *Escherichia coli* tRNA<sup>Tyr</sup> gene clusters in four different transducing bacteriophages, *J. Mol. Biol.* 128: 21-47.

Rossi, J.J. and Landy, A. (1979) Structure and organization of the two tRNA<sup>Tyr</sup> gene clusters on the *Escherichia coli* chromosome, *Cell* 16: 523-534.

Rossi, J.J., Egan, J., Hudson, L. and Landy, A. (1981) The tyrT locus: Termination and processing of a complex transcript. *Cell* **26**: 305-314.

Hudson, L., Rossi, J.J. and Landy, A. (1981) Dual function transcripts specifying tRNA and mRNA. *Nature* 294: 422-427.

Rossi, J.J., Kierzek, R., Huang, T., Walker, P.A. and Itakura, K. (1982) An alternate method for assembling and cloning synthetic genes. *J. Biol. Chem.* **257**: 9226-9229.

Rossi, J.J., Schold, M., Larson, G.P. and Wallace, R.B. (1982) Functional expression of a yeast ochresuppressor tRNA gene in Escherichia coli. Gene 2: 423-432.

Reilly, J.C., Ogden, R. and Rossi, J.J. (1982) Isolation of a mouse pseudo tRNA gene that encodes CCA: A possible example of reverse flow of genetic information. *Nature* **300**: 287-289.

Larson, G.P., Itakura, K., Ito, H. and Rossi, J.J. (1983) Saccharomyces cerevisia actin - Escherichia coli lacZ gene fusions: synthetic-oligonucleotide-mediated deletion of the 309 b.p. intervening sequence in the actin gene. Gene 22: 31-39.

Rossi, J.J., Soberon, X., Marumoto, Y., McMahon, J. and Itakura, K. (1983) Biological expression of an Escherichia coli consensus sequence promoter and some mutant derivatives. *Proc. Natl. Acad. Sci. USA* 80: 3203-3207.

Itakura, K., Rossi, J.J. and Wallace, R.B. (1984) Synthesis and use of synthetic oligonucleotides. *Ann. Rev. Biochem.* 53: 323-356.

Larson, G., Kaplan, B. and Rossi, J.J. (1984) The chemical synthesis of DNA and its uses in molecular biology. *Amer. Biol. Teacher* **46**(8): 440-446.

Ng, R., Domdey, H., Larson, G., Rossi, J.J. and Abelson, J. (1985) Deletion of the intron from the Saccharomyces cerevisiae chromosomal actin gene. Nature 314: 183-184.

Cellini, A., Parker, R., Guthrie, C., and Rossi, J.J. (1986) Activation of a cryptic TACTAAC box in the Saccharomyces cerevisiae actin intron. Molec. and Cell Biol. 6: 571-1578.

Cellini, A., Felder, E. and Rossi, J.J. (1986) Yeast premessenger RNA splicing efficiency depends on critical spacing requirements between the branch point and 3' splice site. *EMBO J.* 5: 1023-1030.

Vijayraghavan, U., Parker, R., Tamm, J., Limura, Y., Rossi, J.J., Abelson, J. and Guthrie, C. (1986) Mutations in conserved intron sequences affect multiple steps in the yeast splicing pathway, particularly assembly of the spliceosome. *EMBO J.* 5: 1683-1695.

Felder, E., Cellini, A. and Rossi, J.J. (1987) Oligonucleotide mediated mutational analysis of recognition sequence effects on lariat formation during messenger RNA splicing in yeast. *Nucleosides and Nucleotides* 6(1&2): 409-411.

Kazuyu, E., Schold, M., Rossi, J.J. and Wallace, R.B. (1987) Enzymatic synthesis of oligoribonucleotides of defined sequence. *DNA* 6: 497-504.

Ouellette, M., Rossi, J.J., Bazin, R. and Roy, P.H. (1987) Oligonucleotide probes for the detection of TEM-1 and TEM-2 b-lactamase genes and their transposons. *Can. J. of Micro.* 33: 205-211.

Murakawa, G.J., Wallace, R.B., Zaia, J.A., Spallone, P.A. and Rossi, J.J. (1988) Direct detection of HIV-1 RNA from AIDS and ARC patient samples. DNA 7(4): 287-295.

Kashani-Sabet, M., Rossi, J.J., Lu, Y. and Scanlon, K.J. (1988) Early detection of drug resistance in human tumors by *in vitro* enzymatic amplification. *Cancer Res.* 48: 5775-5778.

Zaia, J.A., Rossi, J.J., Murakawa, G.J., Spallone, P.A., Stephens, D.A., Kaplan, B.E. Eritja, R., Wallace, R.B. and Cantin, E.M. (1988) Inhibition of human immunodeficiency virus by using an oligonucleotide methylphosphonate targeted to the *tat-*3 gene. *J. Virology* 62: 3914-3917.

Lange, W., Snyder, D.S., Castro, R., Rossi, J.J. and Blume, K.G. (1989) Detection by enzymatic amplification of bcr-abl mRNA in peripheral blood and bone marrow cells of patients with chronic myelogenous leukemia. *Blood* 73: 1735-1741.

Deshler, J.O., Larson, G.P. and Rossi, J.J. (1989) Kluyveromyces lactase maintains Saccharomyces cerevisiae conserved intron sequences. J. Mol. Cellular Biol. 9: 2208-2213.

Holland, H.K., Saral, R., Rossi, J.J., Donnenberg, A.D., Burns, W.H., Beschorner, W.M., Farzadegan, H. Jones, R.J., Quinnan, G.V., Vogelsang, G.B., Vriesendorp, H.M., Wingard, J.R., Zaia, J.A. and Santos, G.W. (1989) Allogeneic bone marrow transplantation, zidovudine, and human immunodeficiency virus type 1 (HIV-1) infection: studies in a patient with non-hodgkin lymphoma. *Ann. of Intern. Med.* 3(12): 973-981.

Scanlon, K.J., Kashani-Sabet, M., Miyachi, H., Sowers, L.C. and Rossi, J.J. (1989) Molecular basis of cisplatin resistance in human carcinomas: Model systems and patients. *Anticancer Res.* 9: 1301-1312.

Sarver, N., Cantin, E., Chang, P., Ladne, P., Stephens, D., Zaia, D., and Rossi, J.J. (1990) Ribozymes as potential anti-HIV-1 therapeutic agents. *Science* **247**: 1222-122.

Chang, P.S., Cantin, E.M., Zaia, J.A., Ladne, P.A., Stephens, D.A., Sarver, N. and Rossi, J.J. (1990) Ribozyme-mediated site-specific cleavage of the HIV-1 genome. *Clin. Biotechnology* 2: 23-31.

Sheibani, K., Ben-Ezra, J., Swartz, W.G., Rossi, J.J., Kezirian, J., Koo, C.H. and Winberg, C.D. (1990) Monocytoid B-cell lymphoma in a patient with acquired immune deficiency syndrome: Demonstration of HIV sequences in paraffin-embedded lymph node sections by polymerase chain reaction amplification. *Archives of Path. & Lab. Med.* 114: 1264-1267.

Mivechi, N.F., and Rossi, J.J. (1990) Use of modified polymerase chain reaction to detect the expression of the 70 kDa heat shock genes in control or heat shocked human leukemia cells as correlated to their heat response, *Cancer Res.* **50**: 2877-2884.

Castanotto, D., and Rossi, J.J. (1991) Can the rate of splicing influence the ratio of two alternatively chosen 3' splice site? J. Cellular Biochem., Supplement 15D, Wiley-Liss, Inc., New York, p.61

Deshler, J. and Rossi, J.J. (1991) Unexpected point mutations activate cryptic 3' splice sites by perturbing a natural secondary structure within a yeast intron. Genes and Develop. 5: 1252-1263.

Ben-Ezra, J., Johnson, D., Rossi, J.J., Cook, N. and Wu, A. (1991) Effect of fixation on the amplification of nucleic acids from paraffin embedded material by the polymerase chain reaction. *J. Histochem. and Cytochem.* **39**: 351-354.

Rossi, J.J., Cantin, E.M., Sarver, N. and Chang, P.S. (1991) The potential use of catalytic RNA's in therapy of HIV infection and other diseases. *Pharmacology and Therapeutics* 50(2): 245-254.

Taylor, N. and Rossi, J.J. (1991) Ribozyme mediated cleavage of HIV-1 GAG RNA: The effects of non-targeted sequences and secondary structure on ribozyme cleavage activity. *Antisense Res. and Development* 1: 173-186.

Larson, G.P. and Rossi, J.J. (1991) Altered response to growth rate changes in *Kluyveromyces lactis* versus *Saccharomyces cerevisiae* as demonstrated by heterologous expression of ribosomal protein 59 (CRY1) *Nucleic Acids Res.* 19: 4701-4707.

Scanlon, K.J., Jiao, L., Funato, T., Wang, W., Tone, T., Rossi, J.J. and Kashani-Sabet, M. (1991) Ribozyme-mediated cleavage of c-fos mRNA reduces gene expression of DNA synthesis enzymes and metallothionein. *Proc. Natl. Acad. Sci. USA* 88: 10591-10595.

Rossi, J.J., Elkins, D., Taylor, N., Zaia, J., Sullivan, S. and Deshler, J.O. (1991) Exploring the use of antisense, enzymatic RNA molecules (ribozymes) as therapeutic agents. *Antisense Res. and Development* 1: 285-288.

Castanotto, D., Rossi, J.J. and Deshler, J.O. (1992) Biological and functional aspects of catalytic RNAs. *Critical Rev. Eukaryotic Gene Exp.* 2: 331-357.

Rossi, J.J., Elkins, D., Zaia, J.A. and Sullivan, S. (1992) Ribozymes as anti-HIV-1 therapeutic agents: Principles, applications and problems. AIDS Research and Human Retroviruses 8: 183-189.

McCarrey, J.R., Berg, W.M., Paragioudakis, S.J., Zhang, P.L., Dilworth, D.D., Arnold, B.L. and Rossi, J.J. (1992) Differential transcription of *PgK* genes during spermatogenesis in the mouse. *Developmental Biol.* 154: 160-168.

Taylor, N.R. Kaplan, B.E. Swiderski, P, Li, H. and Rossi, J.J. (1992) Chimeric DNA-RNA hammerhead ribozymes have enhanced in vitro catalytic efficiency and increased stability in vivo. Nucleic Acids Res. 20: 4559-4565.

Castanotto, D. and Rossi, J.J. (1992) Small sequence insertions within the branch point region dictate alternative sites of lariat formation in a yeast intron. *Nucleic Acids Res.* 20: 6649-6655.

Shimayama, T., Sawata, S. Komiyama, M., Takagi, Y., Tanaka, Y., Wade, A., Sugimoto, N., Rossi, J.J., Nishikawa, F., Nishikawa, S. and Taira, K. (1992) Substitution of non-catalytic stem and loop regions of hammerhead ribozyme with DNA counterparts only increases KM without sacrificing the catalytic step (kcat): A way to improve substrate-specificity. *Nucleic Acids Res. Symp. Ser.* 27: 17-18.

Arnold, B.L., Itakura, K. and Rossi, J.J. (1992) PCR based quantitation of low levels of HIV-1 DNA using an external standard. GATA 9: 113-116.

Snyder, D.S., Wu, Y., Wang, J.L., Rossi, J.J., Swiderski, P., Kaplan, B.E. and Forman, S.J. (1993) Ribozyme-mediated inhibition of bcr-abl gene expression in a Philadelphia chromosome-positive cell line. *Blood* 82: 600-605.

Lisziewicz, J., Sun, D., Smythe, J., Lusso, P., Lori, F., Louie, A., Markham, P., Rossi, J.J., Reitz, M. and Gallo, R.C. (1993) Inhibition of HIV-1 replication by regulated expression of a polymeric-TAR RNA decoy as a strategy for gene therapy. *Proc. Natl. Acad. Sci. USA* 90: 8000-8004.

Natarajan, R., Gu, J-L., Rossi, J.J., Gonzales, N., Lanting, L., Xu, L. and Nadler, J. (1993) Elevated glucose and angiotensin II increase 12-lipoxygenase activity and expression in porcine aortic smooth muscle cells. *Proc. Natl. Acad. Sci. USA* 90: 4947-4951.

Larson, G.P., Bertrand, E. and Rossi, J.J. (1993) Designing, and testing of ribozymes as therapeutic agents. *Methods: A Companion To Methods In Enzymology* 5: 19-27.

Sarver, N. and Rossi, J.J. (1993) Gene therapies for HIV infection: Progress and prospects. *The NIH Journal* 5(9): 63-67.

Dropulic, B., Elkins, D.A., Rossi, J.J. and Sarver, N. (1993) Ribozymes: Use as anti-HIV therapeutic molecules. *Meeting Report Antisense Res. Develop.* 3: 87-94.

Bertrand, E.L. and Rossi, J.J. (1994) Facilitation of hammerhead ribozyme catalysis by the nucleocapsid protein of HIV-1 and the heterogeneous nuclear ribonucleoprotein A1. *EMBO J.* 13: 2904-2912.

Larson, G.P., Castanotto, D., Rossi, J.J. and Malafa, M.P. (1994) Isolation and functional analysis of a Kluyveromyces lactis RAP1 homologue. Gene 150: 35-41.

Castanotto, D., Rossi, J.J. and Sarver, N. (1994) Antisense catalytic RNAs as therapeutic agents. Adv. Pharmacol. 25: 289-317.

Zhou, C., Bahner, L.C., Larson, G.P., Zaia, J.A., Rossi, J.J. and Kohn, D.B. (1994) Inhibition of HIV-1 in human T-lymphocytes by retroviraly transduced Anti-tat and rev hammerhead ribozymes. Gene 49: 33-39.

Rossi, J.J. (1994) Making ribozymes work in cells. Current Biology 4: 469-471.

Gu, J.-L., Natarajan, R., Ben-Ezra, J., Valente, G., Scott, S., Yoshimoto, T., Yamamoto, S., Rossi, J.J. and Nadler, J.L. (1994) Evidence that a leukocyte type of 12-Lipoxygenase is expressed and regulated by angiotensin II in human adrenal glomerulosa cells. *Endocrinology* 134: 70-77.

Deshler, J.O., Li, H., Rossi, J.J. and Castanotto, D. (1995) Ribozymes expressed within the loop of a natural antisense RNA form rho-independent transcriptional terminators. Gene 155: 35-43.

Gu, J.-L., Veerapanane, D., Rossi, J.J., Natarajan, R., Thomas, L. and Nadler, J. (1995) Ribozyme-Mediated Inhibition of Expression of Leukocyte-type 12-Lipoxygenase in Procine Aortic Vascular Smooth Muscle Cells. *Circulation Res.* 77: 14-20.

Heidenreich, O., Kang, S., Brown. D., Xu, X., Swiderski, P., Rossi, J.J., Eckstein, F. and Nerenberg, M. (1995) Ribozyme-mediated RNA degradation in nuclei suspension. *Nucleic Acids Res.* 23(12): 2223-2228.

Rossi, J.J. (1995) Controlled, Targeted, Intracellular Expression of Ribozymes; Progress and Problems. *Trends in Biotechnology* 13: 301-306.

Westaway, S.K., Larson, G.P., Li, S., Zaia, J.A. and Rossi, J.J. (1995) A Chimeric tRNA<sup>lys3</sup>-Ribozyme Inhibits HIV Replication Following Virion Assembly Nucleic Acids Research, Symposium, RNA Biology: RNA Protein Interactions. **33**: 194-199.

Castanotto, D., Bertrand, E. and Rossi, J.J. (1995) Antisense Technology and Ribozymes. *Molecular Biology, Current Innovations and Future Trends* Part 2: 103-114.

Akhtar, S. and Rossi, J.J. (1996) Anti-HIV therapy with antisense oligonucleotides and ribozymes: realistic

approaches or expensive myths? Journal of Antimicrobial Chemotherapy 38: 159-165.

Heidenreich, O., Xu, X., Swiderski, P., Rossi, J.J. and Nerenberg, M., (1996) Correlation of activity with stability of chemically modified ribozymes in nuclei-suspension. *Antisense Res. & Develop.* 6: 111-118.

Zhou. C., Bahner, I., Rossi, J.J. and Kohn D.B. (1996) Expression of Hammerhead Ribozymes by Retroviral Vectors to inhibit HIV-1 Replication: Comparison of RNA Levels and Viral Inhibition. *Antisense Research & Development* 6: 17-24

Lin, J. and Rossi, J.J. (1996) Identification and characterization of yeast mutants that overcome an experimentally introduced block to splicing at the 3' splice site. RNA 2: 835-848.

Gu, J.-L., Nadler, J. and Rossi, J.J. (1997) Use of a hammerhead ribozyme with cationic liposomes to reduce leukocyte 12-lipoxygenase expression in vascular smooth muscle. J. Mol. Cellular Biochem. 172: 47-57.

Good, P.D., Krikos, A.J., Li, S.X., Bertrand, E., Lee, N.-S., Giver, L. Ellington, A., Zaia, J.A. Rossi, J.J. and Engelke, D.R. (1997) Expression of small, therapeutic RNAs in human cell nuclei. *Gene Therapy* 4: 45-54.

Bertrand, E., Castanotto, D., Zhou, C., Carbonnelle, C., Lee, N.-S., Good, P., Chatterjee, S., Grange, T. Pictet, R., Kohn, D. Engelke, D. and Rossi, J.J. (1997) The expression cassette determines the functional activity of ribozymes in mammalian cells by controlling intracellular localization. *RNA* 3: 75-88.

Snyder, D.S., Wu, Y., McMahon, R., Yu, L., Rossi, J.J. and Forman, S.J. (1997) Ribozyme-mediated inhibition of a Philadelphia chromosome-positive acute lymphoblastic leukemia cell line expressing the p190 bcr-abl oncogene. *Biology of Blood and Marrow Transplantation* 3: 179-186.

Castanotto, D. and Rossi. J.J. (1998) Cooperative interaction of branch signals in the actin intron of Saccharomyces cerevisiae. *Nucleic Acids Res.* **26**(18): 4137-4145.

Westaway, S.K., Chang, Z. Cagnon, L., Li, S., Li, H., Larson G.P., Zaia, J.A. and Rossi, J.J. (1998) Virion encapsidation of tRNA3<sup>Lys</sup>-Chimeric RNAs inhibits HIV infection. *Antisense and Nucl. Acid Drug Develop* 8: 185-197.

Kechli, A., Freiden, P.J., Rossi, J.J., Brenner, M.K., Choueiry, M.A., Garcia, J.V. and Slobod, K.S. (1998) Expression of the Human Immunodeficiency Virus Type 1 Primer Binding Sequence Inhibits HIV-1 Replication. *Human Gene Therapy* 9: 587-590.

Rossi, J.J. (1998) Therapeutic Ribozymes: Principles and Applications. BioDrugs 9(1); 1-10.

Castanotto, D., Li, H., Chow, W., Rossi, J.J. and Deshler, J.O. (1998) Structural similarities between hammerhead ribozymes and the spliceosomal RNAs could be responsible for lack of ribozyme cleavage in yeast. *Antisense and Nucl. Acid Drug Develop.* 8: 1-13.

Konopka, K., Rossi, J.J., Swiderski, P., Slepushkin, V.A. and Düzgünes, N. (1998) Delivery of an anti-HIV-1 ribozyme into HIV-infected cells via cationic liposomes. *Biochimica et Biophyscia Acta* 1372: 55-68.

Konopka, K., Düzgünes, N., Rossi, J.J. and Lee, N.-S. (1998) Receptor Ligand-Facilitated Cationic Liposome Delivery of Anti-HIV-1 Rev-Binding Aptamer and Ribozyme DNAs. J. Drug Targeting 5(4): 247-259.

Pal, B.K., Scherer, L., Zelby, L., Bertrand, E. and Rossi, J.J. (1998) Monitoring Retroviral RNA Dimerization in vivo via Hammerhead Ribozyme Cleavage. J. Virol. 72(10): 8349-8353.

Rossi, J.J. (1998) Ribozymes to the rescue: repairing genetically defective mRNAs. *Trends in Genetics* **14**(8): 295-335.

Scherr, M. and Rossi, J.J. (1998) Rapid determination and quantitation of the accessibility to native RNAs by antisense oligodeoxynucleotides in cell extracts. *Nucleic Acids Res.* **26**(22): 5079-5085.

Rossi, J.J. (1999) Ribozymes, Genomics and Therapeutics. Chem. & Biol. 6(2): R33-37.

Xu, Z.-D, Oey, L., Kawachi, M.H., Lee, N.-S., Rossi, J.J. and Fujita-Yamaguchi, Y. (1999) Hammerhead ribozyme-mediated cleavage of the human insulin-like growth factor-II RNA in vitro and in prostate cancer cells. Endocrinology 140(5): 2134-2144.

Castanotto, D., Chow, W.A., Li, H. and Rossi, J.J. (1998) Unusual interactions between cleavage products of a cis-cleaving hammerhead ribozyme. *Antisense Res. & Develop.* 8: 499-506.

Browning, C.M., Cagnon, L., Good, P.D., Rossi, J.J., Engelke, D.R. and Markovitz, D.M. (1999) Potent Inhibition of Human Immunodeficiency Virus Type 1 (HIV-1) Gene Expression Virus Production by an HIV-2 Tat Activation-Response DNA Decoy. J. Viral. 73(6): 5191-5195.

Lee, N.-S., Bertrand, E. and Rossi, J.J. (1999) mRNA localization signals can enhance the intracellular effectiveness of hammerhead ribozymes. RNA 5: 1200-1209.

Rossi, J.J. (1999) Ribozymes in the Nucleolus. Science 285: 1685.

Kim, D.-H. and Rossi, J.J. (1999) The first ATPase domain of the yeast 246-kDa protein is required for in vivo unwinding of the U4/U6 duplex, RNA 5: 959-971.

Kim, D.-H. and Rossi, J.J. (1999) Simple Identification of Mutant Clones. Biotechniques 27(4): 680-681.

Michienzi, A., Cagnon, L., Bozzoni, I. and Rossi, J.J. (1999) A chimeric nucleolar Rev decoy inhibits the HIV replication. *Nucleic Acids Res.* 41: 211-214.

Wu, Y., Yu, L., McMahon, R., Rossi, J.J., Forman, S.J. and Snyder, D.S. (1999) Inhibition of bcr-abl Oncogene Expression by Novel Deoxyribozymes (DNAzymes). *Human Gene Therapy* 10: 2847-2857.

Rossi, J.J. (1999) The application of ribozymes to HIV infection. Curr. Opinion Mol. Therapeutics 1(3): 316-322.

Chen, H.-H., Castanotto, D., LeBon, J.M., Rossi, J.J. and Riggs, A.D. (2000) In vivo, high-resolution analysis of yeast and mammalian RNA-protein interactions, RNA structure, RNA splicing and ribozyme cleavage by use of terminal transferase-dependent PCR. Nucleic Acids Res. 28: 1656-1664.

Bai, J., Gorantla, S., Banda, N., Cagnon, L., Rossi, J.J. and Akkina, R. (2000) Characterization of Anti-CCR5 Ribozyme-Transduced CD34+ Hematopoietic Progenitor Cells in Vitro and in a SCID-hu Mouse Model in Vivo. Mol. Ther. 1(3): 244-254.

Scherr, M., Rossi, J.J., Sczakiel, G. and Patzel, V. (2000) RNA Accessibility Prediction: A Theoretical Approach is Consistent with Experimental Studies in Cell Extracts. *Nucleic Acids Res.* 28(13): 2455-2461.

Michienzi, A., Cagnon, L., Bahner, I. and Rossi, J.J. (2000) Ribozyme-mediated inhibition of HIV 1 suggests nucleolar trafficking of HIV-1 RNA. *Proc. Natl. Acad. Sci. USA* 97(16): 8955-8960.

Scherr, M., Reed, M., Huang, C.-F., Riggs, A.D. and Rossi, J.J. (2000) Oligonucleotide Scanning of Native mRNAs in Extracts Predicts Intracellular Ribozyme Efficiency: Ribozyme-Mediated Reduction of the Murine DNA Methyltransferase. *Mol. Ther.* **2**(1): 26-38.

Cagnon, L. and Rossi, J.J. (2000) Downregulation of the CCR5 β-Chemokine Receptor and Inhibition of HIV-1 Infection by Stable VA1-Ribozyme Chimeric Transcripts. *Antisense & Nucl. Acid Drug. Develop.* 10(4): 251-261.

Konopka, K., Lee, N.-S., Rossi, J.J. and Düzgünes, N. (2000) Rev-binding Aptamer and CMV Promoter Act as Decoys to Inhibit HIV Replication. *Gene* 255: 235-244.

Wilkinson, T.A., Botuyan, M.V., Kaplan, B.E., Rossi, J.J. and Chen, Y. (2000) Arginine Side-chain Dynamics in the HIV-1 Rev-RRE Complex. J. Molecular Biol. 303(4): 515-529.

Rossi, J.J. (2000) Ribozyme therapy for HIV infection. Adv. Drug. Delivery Rev. 44: 71-78.

Lee, N.-S., Sun, B., William. R, Gunkel, N., Salvaterra, P.M. and Rossi, J.J. (2000) Functional co localization of ribozymes and target mRNAs in *Drosophila* occytes. *The FASEB J.* 15: 2303-2554.

Rossi, J.J. (2001) Primate in Utero Gene Transfer Comes of Age. Mol. Ther. 3: 274-275.

Gu, J.-L., Pei, H., Thomas, L., Nadler, J.L., Rossi, J.J., Lanting, L. and Natarajan, R. (2001) Ribozyme-mediated inhibition of rat leukocyte-type 12-lipoxygenase prevents intimal hyperplasia in balloon injured rat carotid arteries. *Circulation* 103: 1446-1452.

Bai, J., Rossi, J.J. and Akkina, R. (2001) Multivalent Anti-CCR5 ribozymes for stem cell-based HIV type 1 gene therapy. *AIDS Res. And Human Retroviruses* 17(5): 385-399.

Patricia, M.-K., Natarajan, R., Dooley, A.M., Hernandez, F., Gu, J.-L., Berliner, J.A., Rossi, J.J., Nadler, J.L., Meidell, R.S., Hedrick, C.C. (2001) Adenoviral Delivery of a leukocyte-type 12 lipoxygenase ribozyme inhibits effects of glucose and PDGF in vascular endothelial and smooth muscle cells. *J. Circulation Res.* 88: 659-665.

Düzgünes, N., Simoes, S., Slepushkin, V., Pretzer, E., Rossi, J.J., DeClercq, E., Antao, V.P., Collins, M.L. and Pedroso de Lima, M.C. (2001) Enhanced inhibition of HIV-1 replication in marcrophages by antisense oligonucleotides, ribozymes and acyclic nucleoside phosphonate analogs delivered in pH-sensitive liposomes. *J. Nucleosides, Nucleotides & Nucl. Acids* 20(4-7): 515-523.

Scherr, M., LeBon, J., Castanotto, D., Cunliffe Schrieb, H., Meltzer, P.S., Ganser, A., Riggs, A.D. and Rossi, J.J. (2001) Detection of antisense and ribozyme accessible-sites on native messenger RNAs: application to amplified in breast cancer-1 (AIB-1) mRNA. *Mol. Ther.* 4: 454-460.

Düzgünes, N., Simoes, S., Konopka, K., Rossi, J.J. and Pedroso de Lima, M.C. (2001) Delivery of novel macromolecular drugs against HIV-1. Expert Opinion Biol. Therapy 1(6): 949-970.

Castanotto, D., Li, H., Krishnan, A., Molina, A., Yam, P., Bauer, G., Kohn, D., Forman, S., Zaia, J. and Rossi, J.J. (2001) RNA based strategies and hematopoietic stem cell gene therapy for HIV infection: progress and future prospects. *Mol. Ther.* 3(5): 922.

Lee, N.-S., Dohjima, T., Bauer, G., Li, H., Li, M.-J., Ehsani, A., Salvaterra, P. and Rossi, J.J. (2002) Expression of small interfering RNAs targeted against HIV-1 rev transcripts in human cells. *Nature Biotech*, 19: 500-505.

Hammond, L., Castanotto, D., Rice, S.R., Nimgaonkar, V.L., Wirshing, D.A., Rossi, J.J., Heston, L.L. and Sobell, J.L. (2002) Alteration of Branch Site Consensus Sequence and Enhanced Pre-mRNA Splicing of an NMDAR1 Intron not Associated with Schizophrenia. *Amer. J. Med. Genetics (Neuropsychiatric Genetics)* 114: 631-636.

Reddy, M.A., Adler, S.G., Kim, Y.-S., Lanting, L., Rossi, J.J., Kang. S.-W., Nadler, J.L., Shahed, A., and Natarajan, R. (2002) Interaction of MAPK and 12-lipoxygenase pathways in growth and matrix protein expression in mesangial cells. *Am. J. Physiol. Renal. Physiol.* 283: F985-F994.

Michienzi, A., Li, S., Zaia, J.A., and Rossi, J.J. (2002) A nucleolar TAR decoy inhibitor of HIV-1 replication. *Proc. Natl. Acad. Sci. USA* 99: 14047-10452.

Chang, Z., Westaway, S., Li, S., Zaia, J.A., Rossi, J.J. and Scherer, L.J. (2002) Enhanced expression and HIV-1 inhibition of chimeric tRNA<sup>Lys3</sup> ribozymes under dual U6 snRNA and tRNA promoters. *Mol. Ther.* 6(4): 481-489.

Castanotto, D., Li, H. and Rossi, J.J. (2002) Functional siRNA expression from transfected PCR products. RNA 8: 1454-1460.

Castanotto, D., Li. J.R., Michienzi, A., Langlois, M.-A., Lee, N.S., Puymirat, J. and Rossi, J.J. (2002) Intracellular ribozyme applications. *Biochem. Soc. Transactions*, 30: 1140-1145.

Bai, J., Banda, N., Rossi, J.J. and Akkina, R. (2002) RNA-Based Anti-HIV-1 Gene Therapeutic Constructs in SCID-hu Mouse Model. Mol. Ther. 6(6): 770-782.

Paul, C.P., Good, P.D., Li, S.X., Kleihauer, A., Rossi, J.J. and Engelke, D.R. (2003) Localized expression of small RNA inhibitors in human cells. *Mol. Ther.* 7(2): 237-247.

Langlois, M.A., Lee, N.S., Rossi, J.J. and Puymirat, J. (2003) Hammerhead Ribozyme-Mediated Destruction of Nuclear Foci in Myotonic Dystrophy Myoblasts, Mol. Ther. 7(5): 670-680.

Michienzi, A., Castanotto, D., Lee, N.-S., Li, S., Zaia, J.A. and Rossi, J.J. (2003) Novel Ribozyme, RNA Decoy, and siRNA Approaches to Inhibition of HIV in a Gene Therapy Setting, *Clin. Appl. Immuno. Rev. CAIR* 3: 223-233.

Dohjima, T., Lee, N.-S., Li, H., Ohno, T. and Rossi, J.J. (2003) Small interfering RNAs (siRNAs) expressed from a Pol III promoter suppress the EWS/Fli-1 transcript in an Ewing's sarcoma cell line. *Mol Ther.* 7: 811-816.

Kim, D.-H. and Rossi, J.J. (2003) Coupling of RNAi mediated target downregulation with gene replacement. *Antisense & Nucl. Acid Drug Develop.* **13**: 151-155.

Banerjea, A., Li, M.-J., Remling, L., Lee, N.-S., Rossi, J.J. and Akkina, R. (2003) Inhibition of HIV-1 by lentiviral vector transduced siRNAs in primary human T-lymphocytes differentiated in SCID-hu mice and CD34+ progenitor cell derived macrophases. *Mol. Therapy* 8: 62-71.

Li, M.-J., Bauer, G., Michienzi, A., Yee, J.-K., Lee, N.-S., Kim, J., Li, S., Castanotto, D., Zaia, J.A. and Rossi, J.J. (2003) Inhibition of HIV-1 Infection by lentiviral vectors expressing Pol III-promoted anti-HIV RNAs. *Mol Ther.* 8: 196-206.

Akkina, R., Banerjea, A., Bai, J., Anderson, J., Li, M.J. and Rossi, J.J. (2003) siRNAs, ribozymes and RNA decoys in modeling stem cell-based gene therapy for HIV/AIDS. *Anticancer Res.* 23: 1997-2005.

Li, M.-J., McMahon, R., Snyder, D.S., Yee, J.-K. and Rossi, J.J. (2003) Specific killing of Ph+ chronic myeloid leukemia cells by a lentiviral vector delivered anti-bcr/abl small hairpin RNA. Oligonucleotides 13(5): 401-409.

Scherer, L.J. and Rossi, J.J. (2003) Approaches for the sequence-specific knockdown of mRNA. *Nature Biotech.* 21(12): 1457-1465.

Michienzi, A., Castanotto, D., Lee, N-S., Li, S., Zaia, J.A., and Rossi, J.J. (2003) RNA-mediated inhibition of HIV in a gene therapy setting. *Ann. N. Y. Acad. Sci.* 1002: 63-71.

Kim, D.-H., Longo, M., Han, Y., Lundberg, P., Cantin, E. and Rossi, J.J. (2004) Interferon induction by siRNAs and ssRNAs by synthesized by phage polymerase. *Nature Biotech.* 22(3): 321-325.

Morris, K.V. and Rossi, J.J. (2004) Anti-HIV-1 Gene Expressing Lentiviral Vectors as an Adjunctive Therapy for HIV-1 Infection. *Current HIV Res.* 2: 185-191.

Dwarakanath, R.S., Sahar, S., Reddy, M.A., Castanotto, D., Rossi, J.J. and Natarajan R. (2004) Regulation of Monocyte Chemoattractant Protein-1 by the oxidized lipid, 13-hydroperoxyoctadecadienoic acid, in Vascular Smooth Muscle Cells via Nuclear Factor-kappa B. (NF-kB), J. Mol. Cell. Cardio. 36: 585-595.

Chen, H.H., Castanotto, D., Le Bon, J., Rossi, J.J. and Riggs, A.D. (2004) In vivo detection of ribozyme cleavage products and RNA structure by use of terminal transferase-dependent PCR. *Methods Mol. Biol.* 252: 109-124.

Lee, N.S. and Rossi, J.J. (2004) Control of HIV-1 Replication by RNA Interference (RNAi), Virus Res. 102: 53-58.

Lu, S., Yu, Q., Binder, G.K., Chen, Z., Slepushkina, T., Rossi, J.J. and Dropulic, B. (2004) Antisense-Mediated Inhibition of Human Immunodeficiency Virus (HIV) Replication by use of an HIV Type 1-Based Vector Results in Severely Attenuated Mutants Incapable of Developing Resistance. *J. Virology* 78: 7079-7088.

Scherer, L.J., Yildiz, Y., Kim, J., Cagnon, L., Heale, B. and Rossi, J.J. (2004) Rapid Assessment of anti-HIV Efficacy using PCR-derived Pol III shRNA cassettes. *Mol. Ther.* **10**(3): 597-603.

Scherer, L.J. and Rossi, J.J. (2004) Recent applications of RNAi in mammalian systems. *Curr. Pharm. Biotechnol.* **5**: 355-360.

Cordelier, P., Kulkowsky, J.W., Ko, C., Matskevitch, A.A., McKee, H.J., Rossi, J.J., Bouhamdan, M., Pomerantz, R.J., Kari, G., Strayer, D.S. (2004) Protecting from R5-tropic HIV: individual and combines effectiveness of a hammerhead ribozyme and a single-chain Fv antibody that targets CCR5. *Gene Ther.* 11: 1627-1637.

Scherer, L.J. and Rossi, J.J. (2004) RNAi applications in mammalian cells. Biotechniques 36: 557-561.

Hannon, G.J. and Rossi, J.J. (2004) Unlocking the potential of the human genome with RNA interference. *Nature* 431: 371-378.

Song, J., Lu, Y.C., Yokoyama, K.K., Rossi, J.J. and Chiu, R. (2004) Cyclophilin A is required for retinoic acid-induced neuronal differentiation in p19 cells. *J. Biol. Chem.* 279: 24424-24429.

Unwalla, H., Li, M.-J., Kim, J.D., Li, H.T., Ehsani, A., Alluin, J. and Rossi, J.J. (2004) Negative feedback inhibition of HIV-1 by TAT-inducible expression of siRNA. *Nature Biotech.* 22: 1573-1578.

Rossi, J.J. (2004) Ribozyme Diagnostics Comes of Age. Chem. Biol. 11: 894-895.

Rossi, J.J. (2004) Medicine: a cholesterol connection in RNAi. Nature 432: 155-156.

Banerjea, A., Li, M.-J., Remling, L., Rossi, J.J. and Akkina, R. (2004) Lentiviral transduction of Tar Decoy and CCR5 ribozyme into CD34+ progenitor cells and derivation of HIV-1 resistant T cells and macrophages. *AIDS Res. and Therapy.* 1; 2.

Scherer, L.J. and Rossi, J.J. (2004) Recent applications of RNA interference (RNAi) in mammalian systems. Letters in Peptide Sci. 10: 255-267.

Gu, S. and Rossi, J.J. (2005) Uncoupling of RNAi from active translation in mammalian cells. RNA 11: 38-44.

Kim, D.-H., Behlke, M.A., Rose, S.D. and Rossi, J.J. (2005) Synthetic dsRNA Dicer substrates enhance RNAi potency and efficacy. *Nature Biotech.* 23: 222-226.

Weinberg, M. and Rossi, J.J. (2005) Comparative single-turnover kinetic analyses of *trans-cleaving* hammerhead ribozymes with naturally derived non-conserved sequence motifs. *FEBS Lett.* **579**: 1619-1624.

Soifer, H.S., Zaragoza, A., Peyvan, M., Behlke, M. and Rossi, J.J. (2005) A potential role for RNA interference in controlling the activity of the human LINE-1 retrotransposon. *Nucl. Acid Res.* **33**(3): 846-856.

Heale, B., Bowers, C., Soifer, H.S. and Rossi, J.J. (2005) siRNA target site secondary structure predictions using local stable substructures. *Nucl. Acid Res.* **33**(3): e30.

Robbins, M.A. and Rossi, J.J. (2005) Sensing the danger in RNA. Nature Med. 11(30): 250-251.

Qian, S., Somlo, G., Zhou, B., Zhu, L., Mi, S., Mo, X., Cheung, E.M., Qiu, W., Lin, R.-J., Rossi, J.J., Holtz, M. Chu, P., Yen, Y. (2005) Ribozyme Cleavage Leads to Decreased Expression of Fibroblast Growth Factor Receptor 3 in Human Multiple Myeloma Cells, Which Is Associated with Apoptosis and Downregulation of Vascular Endothelial Growth Factor. *Oligonucleotides* 15(1): 1-11.

Gonzalez, S., Castanotto, D., Li, H., Olivares, S., Jensen, M.D., Forman, S.J., Rossi, J.J. and Cooper, L.J.N. (2005) Amplification of RNAi-Targeting HLA mRNAs. *Mol. Therapy* 11: 811-818.

Rossi, J.J. (2005) News and Views: Receptor-targeted siRNAs - Protamine-antibody fragment fusions enable delivery of siRNAs to specific cells in mice. *Nature Biotech.* 23: 682-684.

Cook, H.L., Lytle, J.R., Mischo, H.E., Li, M.-J., Rossi, J.J., Silva, D.P., Desrosiers, R.C. and Steitz, J.A. (2005) Small nuclear RNAs encoded by *Herpesvirus saimiri* upregulate the expression of genes linked to T cell activation in virally transformed T cells. *Current Biol.* 15: 974-979.

Castanotto, D., Tommasí, S., Li, M., Li, H., Yanow, S., Pfeifer, G.P. and Rossi, J.J. (2005) Short hairpin RNA directed cytosine (CpG) methylation of the RASSFIA gene promoter in HeLa cells. *Mol. Therapy* 12: 179-183.

Strayer, D.S., Akkina, R., Bunnell, B.A., Dropulic, B., Planelles, V., Pomerantz, R.J., Rossi, J.J. and Zaia, J.A. (2005) Current Status of Gene Therapy Strategies to Treat HIV/AIDS. *Mol. Ther.* 11(6): 823-842.

Rose, S.D., Kim, D-H., Amarzguioui, M., Heidel, J.D., Davis, M.E., Rossi, J.J. and Behlke, M.A. (2005) Functional Polarity is Introduced by Dicer Processing of Short Substrate RNAs. *Nucl. Acid. Res.* **33**: 4140-4156.

Rossi, J.J. (2005) Mammalian Dicer Finds a Partner. EMBO J. 6: 927-929.

Li, M.-J., Kim, J., Li, S., Zaia, J., Yee, J.-K., Anderson, J., Akkina, R. and Rossi, J.J. (2005) Long-Term Inhibition of HIV-1 Infection in Primary Hematopoletic Cells by Lentiviral Vector Delivery of a Triple Combination of Anti-HIV siRNA, Anti-CCR5 Ribozyme and a Nucleolar Localizing TAR Decoy. *Mol. Ther.* 12(5): 900-909.

Langlois, M.-A., Boniface, C., Wang, G., Alluín, J., Salvaterra, P.M., Puymirat, J., Rossi, J.J. and Lee, N.-S. (2005) Cytoplasmic and Nuclear Retained DMPK mRNAs are Targets for RNA Interference in Myotonic Dystrophy Cells. *J. Bio. Chem.* 17: 16949-16954.

Kim, D.-H., Lee, K.-B., Riggs, A.D., Langlois, M.-A., Puymirat, J. and Rossi, J.J. (2005) HnRNP h complex formation on CUG repeat RNAs characteristic of myotonic dystrophy type 1 (DM1). *Nucl. Acid Res.* 33: 3866-3874.

Amarzguioui, M., Rossi, J.J. and Kim, D.-H. (2005) Approaches for chemically synthesized siRNA and vector-mediated RNAi. FEBS Lett. 579: 5974-5981. (One of 25 top downloaded papers for FEBS Letters, 2005)

Unwalla, H., Li, H., Bahner, I., Li, M., Gokhgauzer, Y., Kohn, D. and Rossi, J.J. (2005) Novel Pol II Fusion Promoter Directs Human Immunodeficiency Virus Type 1-Inducible Coexpression of a Short Hairpin RNA and Protein. J. Virol. 80(4): 1863-1873.

Rose, S.D., Kim, D-H., Kim, Amarzguioui, M., Heidel, J.D., Davis, M.E., Rossi, J.J. and Behlke, M.A. (2005) Functional Polarity is Introduced by Dicer Processing of Short Substrate RNAs. *Nucl. Acid. Res.* **33**(13): 4140-4156.

Rossi, J.J. (2005) News and Views - RNAi and the P body connection. Nature Cell Biol. 7: 643-644.

Rossi, J.J. (2006) News and Commentary - A practical siRNA microbicide? Gene Ther. 13: 1493-1494.

Morris, K.V. and Rossi, J.J. (2006) Antiviral applications of RNAi. Cur. Opinion Mol. Therapeutics 8(2): 115-121.

Morris, K.V. and Rossi, J.J. (2006) Lentiviral-mediated delivery of siRNAs for Antiviral Therapy. *Gene Ther.* 13(6): 553-558.

Rossi, J.J. (2006) RNAi as a treatment for HIV-1 infection. Supplement to Biotechniques April, pp. 25-29.

Weinberg, M.S., Villeneuve, Ehsani, A., Amarzguioui, M., Aagaard, L., Chen, A.-X., Riggs, A.D., Rossi, J.J., and Morris, K.V. (2006) The antisense strand of small interfering RNAs directs histone methylation and transcriptional gene silencing in human cells. *RNA* 12(2): 256-262.

Robbins, M.A., Li, M., Leung, I., Li, H., Boyer, D.V., Song, Y., Behlke, M.A. and Rossi, J.J. (2006) Stable Expression of shRNA in human CD34\* progenitor cells can avoid induction of interferon responses to siRNAs in vitro. Nature Biotech. 24: 566-571.

Rossi, J.J. (2006) RNAi therapeutics: SNALPing siRNAs in vivo. Gene Therapy 13(7): 583-584.

Morris, K.V. and Rossi, J.J. (2006) Lentivirus-Mediated RNA Interference Therapy for Human Immunodeficiency Virus Type 1 Infection. *Human Gene Ther.* 17(5): 479-486.

Amarzguioui, M., Lundberg, P., Cantin, E., Hagstrom, J., Behlke, M.A. and Rossi, J.J. (2006) Rational design and in vitro and in vivo delivery of dicer substrate siRNA. *Nature Protoc.* 1(2): 508-517.

Li, M.-J. and Rossi, J.J. (2006) RNAi Based Approaches for the Treatment of HIV Infection. *BIOforum Europe* 10: 34-36.

Unwalla, H. and Rossi, J.J. (2006) RNA interference as a potential antiviral. Future Virology 1(4): 501-508.

Li, M. and Rossi, J.J. (2006) RNAi in Combination with a Ribozyme and TAR Decoy for Treatment of HIV Infection in Hematopoietic Cell Gene Therapy. *Ann. N.Y. Acad, Sci.* 1082: 172-179,

Michienzi, A., Cagnon, L., Kim, J.D., De Angelis, F., Bozzoni, I. and Rossi, J.J. (2006) A nucleolar localizing Rev binding element inhibits HIV replication. *AIDS Res. and Ther.* 3: 13.

Kim, D., Villeneuve, L., Morris, K. and Rossi, J.J. (2006) Argonaute-1 directs siRNA-mediated transcriptional gene silencing in human cells. *Nature Struct. & Mol. Biol.* 13(9): 793-797.

Snove, O. and Rossi, J.J. (2006) Toxicity in mice expressing short hairpin RNAs gives a new insight into RNAi. *Genome Biol.* **7**(8): 231.

Snove, O. and Rossi, J.J. (2006) Expressing short hairpin RNAs in vivo. *Nature Methods Perspective* **3**(9): 689-695.

Harris, S. and Rossi, J.J. (2006) Small interfering RNAs to the rescue: blocking L1 retrotransposition. *Nature Struct. & Mol. Biol.* 13(9): 758-759.

Snove, O. and Rossi, J.J. (2006) Chemical Modifications Rescue Off-Target Effects of RNAi. ACS Chemical Biol. 1(5): 274-276.

Benimetskaya, L., Ayyanar, K., Kornblum, N., Castanotto, D., Rossi, J.J., Wu, S., Lai, J., Brown, B., Popova, N., Miller, P., McMicken, H., Chen, Y. and Stein, C.A. (2006) The Function of Bcl-2 Protein in 518A2 Melanoma Cells In Vivo Differs Dramatically from its In Vitro Role. Clin. Cancer Res. 12(16): 4940-4948.

Gu, S., Ji, J., Kim, J.D., Yee, J.-K., and Rossi, J.J. (2006) Inhibition of infectious HIV-1 virions via lentiviral vector encoded short antisense RNAs. *Oligonucleotides* 16: 287-295.

Paul, S., Dansithong, D., Kim, D., Rossi, J.J., Webster, N., Comai, L. and Reddy, S. (2006) Interaction of muslebland, CUG-BP-GP1 and hnRNP H proteins in DM-1 associated aberrant IR splicing. *EMBO J.* 25: (18): 4271-4283.

Stein, C. and Rossi, J.J. (2006) A critical assessment of the potential of short interfering RNA therapeutics. *Drug Discovery Today* 2: 27-31.

Kolb, G., Reigadas, S., Castanotto, D., Ventura, M., Rossi, J.J. and Toulme, J.-J. (2006) Endogenous Expression of an Anti-TAR Aptamer Reduces HIV-1 Replication. RNA Biol. 3(4): 150-156.

Rossi, J.J. (2006) Commentary - Partnering aptamer and RNAi technologies. Mol. Ther. 14(4): 461-462.

Unwalla, H.J. and Rossi, J.J. (2006) Promoting Gene Therapy: Expression Systems for Transgenes and Post-transcriptional Gene Silencing. Biotechnol. & Genetic Engineering Rev. 23: 71–91.

Li, N., Wang., Y., Forbes, K., Vignali, K.M., Heale, B.S., Hartmann, D., Black, R.A., Rossi, J.J., Blobel, C.P., Dempsey, P.J., Workman, C.J., Vignali, D.A.A. (2007) Metalloproteases regulate T-cell proliferation and effector function via LAG-3. *EMBO J.* 26(20): 494-504.

Rossi, J.J. (2007) News and Views - Transcriptional activation by small RNA duplexes. *Nat. Chem. Biol.* **3**(3): 136-137.

Kim, D.H. and Rossi, J.J. (2007) Strategies for silencing human disease using RNA interference, Nat. Rev. Genetics 8(3): 173-184.

Saetrom, P., Heale, B., Snove, O. Aagaard, L. and Rossi, J.J. (2007) Distance constraints between microRNA target sites dictate efficacy and cooperativity. *Nucl. Acids Res.* **35**(7): 2333-2342.

Heidel, J.D., Liu, J.Y.-C., Yen, Y., Zhou, B., Heale, B.S.E., Rossi, J.J., Bartlett, D.W. and Davis, M.E. (2007) Potent siRNA Inhibitors of Ribonucleotide Reductase Subunit RRM2 Reduce Cell Proliferation *In vitro* and *In vivo. Clin. Cancer Res.* 13(7): 2207-2215.

Aagaard, L., Amarzguioui, M., Sun, G., Santos, L.C., Ehsani, A., Prydz, H. and Rossi J.J. (2007) A Facile Lentiviral Vector System for Expression of Doxycycline-Inducible shRNAs: Knockdown of the Pre-miRNA Processing Enzyme Drosha. *Mol. Ther.* 15(5): 938-945.

Scherer, L., Frank, R. and Rossi, J.J. (2007) Optimization and Characterization of tRNA-shRNA Expression Constructs. *Nucl. Acids Res.* 35(8): 2620-2628.

Anderson, J., Li, M.-J., Palmer, B., Remling, L., Li, S., Yam, P., Yee, J.-K., Rossi, J.J., Zaia, J. and Akkina, R. (2007) Safety and Efficacy of a Lentiviral Vector Containing Three Anti-HIV Genes-CCR5 Ribozyme, Tatrev siRNA, and TAR Decoy in SCID-hu mouse-Derived T Cells. *Mol. Ther.* 15: 1182-1188.

Aagaard, L, and Rossi, J.J. (2007) RNAi Therapeutics: Principles, Prospects and Challenges. Adv. Drug Delivery Rev. 59: 75-86.

Saetrom, P., Snove, O. and Rossi, J.J. (2007) Epigenetics and MicroRNAs. Pediatric Res. 61(5 Pt 2): 17R-23R.

Kim, J.-W., Zhang, Y.-H., Zern, M.A., Rossi, J.J. and Wu, J. (2007) Short hairpin RNA causes the methylation of transforming growth factor-β receptor II promoter and silencing of the target gene in rat hepatic stellate cells. *Biochem. & Biophy, Res. Comm.* 359: 292-297.

Scherer, L., Rossi, J.J. and Weinberg, W.S. (2007) Review, Progress and Prospects: RNA-based therapies for treatment of HIV infection. Gene Ther. 14(14): \$1057-1064.

Cantin, E. M. and Rossi, J.J. (2007) News and Views: Molecular Medicine - Entry Granted. *Nature* 448: 33-34.

Castanotto, D., Sakurai, K., Lingeman, R., Li, H., Shively, L., Aagaard, L., Soifer, H., Gatignol, A., Riggs, A. and Rossi, J.J. (2007) Combinatorial delivery of small interfering RNAs reduces RNAi efficacy by selective incorporation into RISC. *Nucl. Acids Res.* 35(15): 5154-5164.

Rossi, J.J. (2007) Targeted cleavage: Tuneable cis-cleaving ribozymes. Proc. Natl. Acad. Sci. USA 104: 14881-14882.

Kato, M., Zhang, J., Wang, M., Lanting, L., Yuan, H., Rossi, J.J. and Natarajan, R. (2007) MicroRNA-192 in Diabetic Kidney Glomeruli and its Function in TGF-beta-induced Collagen Expression via Inhibition of E-box Repressors. *Proc. Natl. Acad. Sci. USA* 104(9): 3432-3437.

Held, D.M., Kissel, J.D., Thacker, S.J., Michalowski, D., Saran, D., Ji, J. Hardy, R.W., Rossi, J.J. and Burke, D.H. (2007) Cross-clade inhibition of recombinant HIV-1 HIV-2 and SIVcpz reverse transcriptases by RNA pseudoknot aptamers. J. Virol. 81(10): 5375-5374.

Rossi, J.J., June, C.H. and Kohn, D.B. (2007) Review - Genetic therapies against HIV. Nat. Biotech. 25(12): 1-11.

Soifer, H., Rossi, J.J. and Saetrom, P. (2007) MicroRNAs in disease and potential therapeutic applications. *Mol. Ther.* 15: 2070-2079.

Sano, M., Li, H., Nakanishi, M. and Rossi, J.J. (2008) Expression of Long Anti-HIV1 Hairpin RNAs for the Generation of Multiple siRNAs: Advantages and Limitations. *Mol. Ther.* 16: 170-177.

Zhou, J., Li, H., Li, S., Zaia, J. and Rossi J.J. (2008) Novel dual inhibitory function aptamer-siRNA delivery system for HIV-1 therapy. *Mol. Ther.* 16(8): 1481 – 1489.

Unwalla, H and Rossi J.J. (2008) Tat-regulated Expression of RNA Interference: Triggers for the Treatment of HIV Infection. Current HIV/AIDS Reports 5(1): 40-43.

Collingwood, M.A., Rose, S.D., Huang, L., Hillier, C., Amarzguioui, M., Wilger, M.T., Soifer, H.S., Rossi, J.J. and Behlke, M.A. (2008) Chemical Modification Patterns Compatible with High Potency Dicer-Substrate Small Interfering RNAs. *Oligonucleotides* 18: 187-199.

Zhou, J. and Rossi, J.J. (2008) Bivalent Aptamers Deliver the Punch. Chem. & Biol. 15 644-645.

Rossi, J.J. (2008) Expression Strategies for Short Hairpin RNA Interference Triggers. *Hum. Gene Ther.* 19(4): 313-317.

Lee, N.-S., Kim, D.H., Alluin, J., Robbins, M., Gu, S., Li, H., Kim, J., Salvaterra, P.M. and Rossi J.J. (2008) Functional and intracellular localization properties of U6 promoter-expressed siRNAs, shRNAs, and chimeric VA1 shRNAs in mammalian cells. RNA 14(9): 1823-1833.

Kim, D. and Rossi J.J. (2008) RNAi Mechanisms and Applications. BioTechniques 44(5): 613-616.

Aagaard, L., Zhang, J., von Eije, K.J., Li, Haitang, Saetrom, P., Amarzguloui, M., and Rossi J.J. (2008) Engineering and optimization of the mir-106b cluster for ectopic expression of multiplexed anti-HIV RNAs. *Gene Ther.* 15:1536-1549.

Kim, D.K., Saetrom, P., Snove, O. and Rossi, J.J. (2008) MicroRNA-directed transcriptional silencing of the cell cycle gene POLR3D. *PNAS Award: Cozzarelli Prize as an exceptional paper published in 2008, Proc. Natl. Acad. Sci. USA* 105: 16230-16235.

Unwalla, H., Li, H., Li, S.-Y., Abad, D. and Rossi J.J. (2008) Use of a U16 sno RNA containing ribozyme library to identify ribozyme targets in HIV. *Mol. Ther.* 16: 1113-1119.

Soifer, H.S., Sano, M., Sakurai, K., Chomchan, P., Saetrom, P., Sherman, M.A., Collingwood, M.A. Behlke, M.A. and Rossi J.J. (2008) A role for the Dicer helicase domain in the processing of thermodynamically unstable hairpin RNAs. *Nucl. Acid. Res.* **36**(20): 6511-6522.

Rossi, J.J. (2008) Realizing the Promise of RNAi. Mol. Ther. 16(5): 810-811.

Castanotto, D. and Rossi, J.J. (2008) The promises and pitfalls of RNAi-based therapeutics. *Nat. Review Insight* **457**: 1-8.

Rossi, J.J. (2009) Cholesterol paves the way for topically applied viricides. Cell Host & Microbe 5: 6-7.

Zhou, J., Swiderski, P., Li, H., Zhang, J., Neff, C.P., Akkina, R., Rossi, J.J. (2009) Selection, characterization ad application of new RNA HIV gp 120 aptamers for facile delivery Dicer substrate siRNAs into HIV infected cells. *Nucleic Acids Res.* Adv. access published March 21, 2009, P. 1-16.

Sakurai, K., Amarzguioui, M., Kim, D.-H., Heale, B., Gatignol, A., Behlke, M.A. and Rossi, J.J. (2008) Human Dicer/TRBP heterodimer selects effective siRNAs and constitutes the RNAi initiation complex. RNA (Submitted)

Ehsani, A., Saetrom, P., Li, H., Snove, L., Aagaard, L. and Rossi, J.J. (2008) Rational design of microRNA-like bi-functional siRNAs targeting HIV and the HIV co-receptor CCR5. *Nat. Biotech.* (Submitted)

Trobridge, G.D., Wu, R., Beard, B.C., Chiu, S.-Y., Munoz, N., Carter, M., von Laer, D., Rossi, J.J. and H.-P. Kiem (2008) Ex Vivo protection from SHIV infection following hematopoietic stem cell gene therapy for AIDS in the pigtailed macaque. *Blood J.* (Submitted)

Kiem, H.P., Wu, R.A., Sun, G., Rossi, J.J. and Trobridge, G.D. (2009) Foamy combinatorial anti-HIV vectors with MGMTP140K potently inhibit HIV-1 and SHIV replication and medicate selection in vivo. *Mol. Therapy* (Submitted)

Sun, G., Yan, J., Noltner, K., Feng, J., Li, H., Sarkis, D.A., Sommer, S.S. and Rossi, J.J. (2009) SNPs in human miRNA genes affect biogenesis and function. RNA J. (Submitted)

Feng, J., Sun, G., Yan, J., Noltner, K., Li, W., Longmate, J., L.L. Heston, Rossi, J.J. and Sommer, S. (2009) Evidence for X-chromosomal schizophrenia associated with microRNA alterations. Pub. Library of Sci. (PLoS 1) (In Press)

Kato, M. Putta, S., Wang, M., Yuan, H., Lanting, L., Nair, I., Gunn, A., Nakagawa, Y., Shimano, H., Todorov, I. Rossi, J.J. and Natarajan, R. (2009) TGF-β activates Akt kinase via a microRNA-dependent amplifying circuit targeting PTEN. *Nature Cell Biol.* (In Press)

Rossi, J.J. (2008) RNAi medicated transcriptional and post-transcriptional regulation of gene expression. *Euro. Pharm. Rev.* (In Press)

Rossi, J.J. (2008) RNAi misses its target. Nat. Med. (In Press).

Kim, D. and Rossi, J.J. (2008) Look Before You Leap. World Pharmaceutical Frontiers (In Press)

Rossi, J.J. (2008) Cholesterol paves the Way for Topically Applied Viricides. *Cell Host and Microbe Press* (In Press)

**Rossi**, J.J. (2009) Innate immunity confounds the clinical efficacy of small interfering RNAs (siRNAs). *Gene Ther.* (In Press)

Rossi, J.J. (2009) Dotting the I's and Crossing the T's: Integration Analyses in Transduced Patient T Cells. *Mol. Ther.* (In Press)

Tiemann, K. and **Rossi**, J.J. (2009) RNAi based therapeutics-current status, challenges and prospects. *EMBO Molecular Medicine* (In Press)

## **BOOK CHAPTERS and PROCEEDINGS**

Rossi, J.J., Egan, J., Berman, M. and Landy, A. (1980) In *Transfer RNA: Biological Aspects*, Chapter 2, pp. 221-224, (Eds., Abelson, P. Schimell and D. Soll), Cold Spring Harbor, New York, NY. The *Escherichia coli* tRNATyr gene clusters: Organization and structure.

Rossi, J.J., Egan, J., Berman, M. and Landy, A. (1980) In Genetics and Evolution of RNA Polymerase, tRNA and Ribosomes, pp. 185-208, (Eds., S. Osawa, H. Ozeki, H. Uchida and T. Yura) University of Tokyo Press, Japan. The Escherichia coli tRNA Tyr gene clusters: Organization, evolution and transcription.

Soberon, X., Rossi, J.J., Larson, G.P. and Itakura, K. (1982) In *Symposium on Promoters: Structure and Function*, pp. 407-234, (Eds., R. Rodriquez and M. Chamberline) Prager Publishers, New York, NY. A synthetic, consensus sequence, prokaryotic promoter is functional.

Rossi, J.J., Kiered, R., Huang, T., Walker, P. and Itakura, K. (1982) In From Gene to Protein: Translation into Biotechnology, pp. 213-234, Academic Press, New York, NY. The role of synthetic DNA in the preparation of structural genes coding for proteins.

Rossi, J.J. and Zoller, M. (1986) In *Protein Engineering*, pp. 51-63, Allan R. Liss, Inc., New York NY. Site specific and regionally directed mutagenesis of protein encoding sequences.

Rossi, J.J. (1987) In *Molecular Biology Reports*, Vol. 1, No. 2, pp. 3-4, Bio-Rad Laboratories, Richmond, CA. RNA probe detection of RNA, alkaline blotted onto Zeta-Probe membranes.

Deshler, J., Kaplan, B., Larson, G. and Rossi, J.J. (1990) In Methods in Nucleic Acids Research, pp. 21-44, (Eds., J.D. Karam, L. Chao and G.W. Warr), CRC Press Inc., Boca Raton, FL. The chemical synthesis and some uses of oligonucleotides in biological research.

Sarver, J., Hampel, A., Cantin, E.M., Zaia, J.A., Chang, P.S., Johnston, M.I., McGowan, J. and Rossi, J.J. (1990) In AIDS: Anti-HIV Agents, Therapies, and Vaccines, pp. 606-609, (Eds., V. St. Georgiev and J. McGowan), New York Academy of Sciences, New York, NY. Self-cleaving RNAs (ribozymes) as new modalities for anti-HIV therapy.

Rossi, J.J., Cantin, E.M., Zaia, J.A., Ladne, P.A., Chen, J., Stephens, D.A., Sarver, N. and Chang, P.S. (1990) In AIDS: Anti-HIV Agents, Therapies, and Vaccines, pp. 184-200, (Eds., V. St. Georgiev and J. McGowan), New York Academy of Sciences, New York, NY. Ribozymes as Therapies for AIDS.

Zaia, J.A., Cantin, E.M., Chang, P.S., Sarver, N. and Rossi, J.J. (1991) In Advances in Molecular Biology and Targeted Treatments for AIDS, pp. 335-343, (Ed., A. Kumar) Plenum Publishing Corp., N.Y. Antiviral evaluation of HIV-1 specific ribozyme expressed in CD4-HELA cells.

Rossi, J.J. and Sarver, N. (1991) In Innovations in Antiviral Development and the Detection of Virus Infections, pp. 95-109, (Ed., L. Walsh) Plenum Publishing Corp., New York, NY. Catalytic antisense RNA (ribozymes): Their potential and use as Anti-HIV-1 therapeutic agents.

Sarver, N. and Rossi, J.J. (1991) In 5e Colloque Des Cent Gardes: Retroviruses of Human A.I.D.S. and Related Animal Diseases, pp. 291-298, (Eds., M. Girard and L. Valette), Marnes-La-Coquette, Paris, France, Catalytic RNAs (ribozymes): A unique anti-HIV strategy.

Rossi, J.J. and Arnold, B. (1991) In Virus Information Exchange Newsletter, pp. 9-11, (Eds., R.J. Coelen) University of Western Australia, Nedlands, Western Australia. The Use of PCR in Viral Diagnosis.

Parker, R., Muhlrad, D., Deshler, J.O., Taylor, N. and Rossi, J.J. (1991) In Gene Regulation Biology of Antisense RNA and DNA, Molecular and Cellular Biology Series, Vol. 1, pp. 55-70, (Eds., Erickson and Izant) Raven Press, Inc., New York, NY. Ribozymes: Principles and designs for their use as antisense and therapeutic agents.

Rossi, J.J. (1992) In Current Opinion in Biotechnology, Vol. 3, (Eds., K. Mosbach, S. Birnbaum and B. Wallace) Current Biology Ltd., London, United Kingdom. Ribozymes.

Zaía, J.A., Chatterjee, S., Wong, K.K., Elkins, D., Taylor, N.R. and Rossi, J.J. (1992) In Antisense Strategies, pp. 95-106, (Eds., R. Baserga and D.T. Denhardt), Ann. NY Acad. Sci., New York, NY. Status of ribozyme and antisense-based developmental approaches for anti-HIV-1 therapy.

Sarver, N., Zaia, J.A. and Rossi, J.J. (1992) In *Annual Review of AIDS Research*, Vol. II, pp. 259-285, (Eds. Koff, Kennedy and Wong-Staal) Marcel-Deckker, Inc., New York, NY. Catalytic RNAs (Ribozymes): A new frontier in biomedical applications.

Deshler, J.O. and Rossi, J.J. (1992) In Nucleic Acid Targeted Drug Design, pp. 557-577, (Eds. T.J. Perun and C.L. Propst) Marcel Dekker, Inc., New York, NY. Catalytic Antisense RNAs: Principles and Design.

Elkins, D.A. and Rossi, J.J. (1995) In Delivery systems for antisense oligonucleotide therapeutics, pp. 17-38, (Ed. S. Akhtar) CRC Press Inc., Boca Raton, FL. Cellular delivery of ribozymes.

Rossi, J.J. (1995) In *British Medical Bulletin*, Gene Therapy Issue, Vol. 51, No. 1., pp. 217-225, (Eds., Goodfellow and Lever) Churchill Livingstone Medical Journals, Edinburgh, U.K. Therapeutic Antisense.

Elkins, D. and Rossi, J.J. (1995) In Delivery Systems for Antisense Oligonucleotide Therapeutics, pp. 17-38, (Ed., S. Akhtar) CRC Press, Inc., Boca Raton, FL. Cellular Delivery of Ribozymes

Castanotto, D., Bertrand, E. and Rossi, J.J. (1995) In HIV, A Practical Approach, Vol. 2, pp. 289-303, (Eds., J. Karn) IRL Press, Oxford, UK. Inhibition of HIV by ribozymes and antisense oligonucleotides, Biochemistry, Molecular Biology and Drug Discovery.

Bertrand, E. and Rossi, J.J. (1996) In *Nucleic Acids & Molecular Biology*, Vol. 10, pp. 301-313, (Eds., F. Eckstein, and D. Lilley) Springer-Verlag, New York, NY. Anti-HIV Hammerhead Ribozymes: Targeting Strategies and Optimization of Intracellular Function.

Rossi, J.J., Bertrand, E. and Castanotto, D. (1996) In *Methods in Molecular Genetics*, Vol. 8, pp. 348-360, (Eds. K. Adolph) Academic Press, San Diego, CA. Ribozymes as Therapeutic Agents and Tools for Gene Analysis.

Lin, R.-J., Kim, D.-H., Castanotto, D., Westaway, S. and Rossi, J.J. (1996) In A Laboratory Guide to RNA: Isolation, Analysis, and Synthesis, pp. 43-50, (Ed., P.A. Krieg) Wiley-Liss, Inc., New York, NY. RNA preparations from yeast cells.

Rossi, J.J., Lee, N.-S., Bertrand, E., Westaway, S., Zhou, C., Bahner, I., Kohn, D., Engelke, D., Good, P., Li, S. and Zaia, J. (1996) In *Journal of Clinical Biochemistry and Nutrition*, Vol. 22, pp. 69-74, (Ed., K. Yagi) Daishowa

Printing Co., Ltd., Tokyo, Japan. Ribozyme therapeutics for the treatment of HIV infection: Progress and future directions.

Lee, N.-S., Bertrand, E. and Rossi, J.J. (1997) In Methods in Molecular Biology, Ribozyme Protocols, Vol. 74, pp. 275-279, (Ed., P. Turner) Humana Press, Totowa, NJ. Enhancement of ribozyme function by RNA binding proteins.

Castanotto, D., Bertrand, E. and Rossi, J.J. (1997) In *Methods in Molecular Biology*, Ribozyme Protocols, Vol. 74, pp. 429-439, (Ed., P. Turner) Humana Press, Totowa, NJ. Exogenous cellular delivery of ribozymes and ribozyme encoding DNAs.

Rossi, J.J. and Sarver, N. (1997) In *Encyclopedia of Cancer*, Vol. III, pp. 1591-1599, (Ed. J.R. Bertino), Academic Press Inc., San Diego, CA. Ribozymes and their applications.

Cagnon, L. and Rossi, J.J. (1997) In Methods in Molecular Biology, Ribozyme Protocols, Vol. 74, pp. 451-457, (Ed., P. Turner) Humana Press, Totowa, NJ. Retroviral delivery and Anti-HIV testing of hammerhead ribozymes.

Rossi, J.J. (1997) In Oligonucleotides as Therapeutic Agents, Ciba Foundation Symposium 209, pp. 195-206, (Ed., Cardew) J. Wiley & Sons, New York, NY. Therapeutic application of catalytic antisense RNAs (ribozymes).

Rossi, J.J. (1998) In Applied Antisense Oligonucleotide Technology, Chapter 28, pp. 511-525, (Eds., C.A. Stein and A.M. Krieg) Academic Press, San Diego, CA. Therapeutic Ribozymes: Principles, applications and problems.

Rossi, J.J. and Forman, S.J. (1998) In Hematopoietic Cell Transplantation, Vol. II, Chapter 22, pp. 225-247, (Eds., E.D. Thomas, K.G. Blume, S.J. Forman) Blackwell Science Inc., Malden, MA. Molecular Inhibition of Gene Expression in Hematopoietic Cells.

Castanotto, D., Scherr, M. and Rossi, J.J. (1999) In Methods in Enzymology, Antisense Technology, Vol. 313, pp. 401-420, (Ed., M. I. Phillips) Academic Press, San Diego, CA. Intracellular Expression and Function of Antisense Catalytic RNA's.

Chen, H.H., Castanotto, D., Rossi, J.J. and Riggs, A.D. (1999) In Intracellular Ribozyme Applications: Principles and Protocols, pp. 217-229, (Eds., J.J. Rossi and L. Couture) Horizon Scientific Press, Norfolk, U.K. RNA analysis by terminal transferase-dependent PCR.

Scherr, M., LeBon, J., Riggs, A.D. and Rossi, J.J. (1999) In Intracellular Ribozyme Applications: Principles and Protocols, pp. 47-56, (Eds., J.J. Rossi and L. Couture) Horizon Scientific Press, Norfolk, U.K. The use of cell extracts and antisense deoxyribo-oligonucleotides for identifying ribozyme cleavage sites on messenger RNAs.

Yamaguchi, Y.F., Li, S.L., Xu, Z.D, Wu, A.M. and Rossi, J.J. (1999) In Proceedings, pp. 99-102, 11th International Conference of Women Engineers and Scientist, Chiba, Japan. Application of biotechnology to the treatment of cancer

Castanotto, D., Scherr, M., Lee, N.-S. and Rossi, J.J. (2000) In *Biotechniques*, pp. 421-440, (Eds., G. Krupp and R. Gaur) Eaton Publications, Natick, MA. Targeting and Intracellular Expression Strategies for Therapeutic Ribozymes.

Michienzi, A. and Rossi, J.J. (2001) In Methods in Enzymology - "Ribonucleases", Vol. 341, pp. 581-596, (Ed., A.W. Nicholson) Academic Press, Inc., San Diego, CA. Intracellular applications of ribozymes.

Rossi, J.J. (2001) In Antisense Drug Technology, pp. 887-906 (Ed., S.T. Cooke) Marcel Dekker, Inc., New York, NY. Intracellular Ribozyme Applications.

Poggi, A. and Rossi, J.J. (2001) In *Encyclopedia of Physical Science and Technology*, Third Edition, Vol. 14, pp. 253-261, (Ed., T.E. Creighton) Academic Press, Inc., San Diego, CA. Ribozymes.

Rossi, J.J. (2002) In Encyclopedia of Molecular Medicine, Vol. 5, pp. 2825-2828, (Ed., T.E. Creighton) John Wiley & Sons, Inc., New York, NY. Ribozymes.

Poggi, A., Michienzi, A. and Rossi, J.J. (2002) In *Pharmaceutical Perspectives of Nucleic Acid-Based Therapeutics*, pp. 54-72, (Eds., R.I. Mahato and S.W. Kim) Taylor and Francis Publishers, London, U.K. Ribozymes as Therapeutic Agents and Genetics Tools.

Rossi, J.J. (2002) In Scientific American, August, pp. 19-22, Killing the Messenger.

Castanotto, D. and Rossi, J. J. (2003) In RNA Interference (RNAi): Nuts and Bolts of RNAi Technology, pp. 189-203, (Eds., D. Engelke) DNA Press, Eagleville, PA. Downregulation of cellular genes by PCR products expressing siRNAs in Mammalian Cells.

Rossi, J.J. (2004) In Cancer Drug Discovery and Development, pp. 45-64, (Eds., A.M. Gewirtz) Humana Press Inc., Totowa, NJ. Therapeutic applications of ribozymes.

Castanotto, D. and Rossi, J.J. (2004) In Methods in Molecular Biology, Vol. 252, pp. 509-514, (Eds., M. Sioud) Humana Press Inc., Totowa, NJ. Construction and Transfection of PCR Products Expressing siRNAs or shRNAs in Mammalian Cells.

Scherer, L.J. and Rossi, J.J. (2004) In Contemporary Cancer Research Cancer Gene Therapy, Chapter 5, pp. 51-63, (Eds., D.T. Curiel and J.T. Douglas) Humana Press Inc. Totowa, NJ. Cancer Therapeutic Applications of Ribozymes and RNAi.

Zaia, J.J., Cairns, J.S. and Rossi, J.J. (2004) In *Hematopoietic Cell Transplantation*, Chapter 100, pp. 1369-1384, (Eds., E.D. Thomas, K.G. Blume, SJ. Forman) Blackwell Science Inc., Malden, MA. AIDS and Hematopoietic Transplantation: HIV Infection, AIDS Lymphoma, and Gene Therapy.

Scherer, L.J. and Rossi, J.J. (2004) In Advances in Genetics, Vol. 52, pp. 1-21, (Eds., J.C. Hall, J.C. Dunlap and T. Friedmann) Elsevier Inc., San Diego, CA. Therapeutic applications of RNA interference: recent advances in siRNA design.

Li, M.-J. and Rossi, J.J. (2005) In *Methods in Enzymology, RNA Interference*, Vol. 392, pp. 218-226, (Eds., D.R. Engelke and J.J. Rossi) Elsevier Inc., San Diego, CA. Lentiviral Vector Delivery of Recombinant Small Interfering RNA Expression Cassettes.

Kim, D.-H., Behlke, M.A., Rose, S.D., Chang, M.-S. Choi, S. and Rossi, J.J. (2005) In *Non-viral Gene Therapy*: Gene Design and Delivery, pp. 465-475 (Eds., K. Taira, K. Kataoka, I, Niidome) Springer-Verlag Publishers, Tokyo, Japan. Enhancing RNAi with synthetic RNA duplexes.

Scherer, L. and Rossi, J.J. (2005) In RNA Interference Technology, From Basic Science to Drug Development, Chapter 29, pp. 406-415, (Ed., K. Appasani) Cambridge University Press, Cambridge, UK. RNAI applications in living animal systems.

Scherer, L.J. and **Rossi**, J.J. (2006) In *Peptide Nucleic Acids, Morpholinos and Related Antisense Biomolecules*. Chapter 8, pp. 133-147 (Eds., C.G. Janson and M.J. During) Kluwer Academic/Plenum Publishers, the Netherlands. Recent Applications of RNA Interference (RNAi) in Mammalian Systems.

Morris, K.V. and Rossi, J.J. (2006) In RNA Towards Medicine, Handbook of Experimental Pharmacology, Vol. 173, pp. 105-116, (Eds., V.A. Erdmann, J. Brosius and J. Barciszewski) Springer-Verlag, Berlin, Heidelberg, Germany. Antiviral Applications of RNAi.

Gaur, R.K. and Rossi, J.J. (2006) Supplement to Biotechniques April, pp. 3-4., (Eds., R.K. Gaur and J.J. Rossi) Biotechniques, Westborough, MA. Introduction: The diversity of RNAi and its applications.

Gaur, R.K. and Rossi, J.J. (2006) Supplement to Biotechniques April, pp. 25-27., (Eds., R.K. Gaur and J.J. Rossi) Biotechniques, Westborough, MA. RNAI as a treatment for HIV-1 infection.

Morris, K.V. and Rossi, J. (2006) In Modern Biopharmaceuticals – Design, Development and Optimization, Vol. 8, pp. 569-580, (Eds., J. Knablein and R.H. Muller) Wiley-VCH Verlag GmbH and Co KgaA, Weinheim, Germany. Combinatorial RNA-based Therapies for HIV-1.

Li, M.-J. and Rossi, J.J. (2006) In Gene Transfer, Delivery and Expression of DNA and RNA, A Laboratory Manual, Chapter 7, pp. 75-82, (Eds., R. Friedmann and J.J. Rossi) Cold Spring Harbor Laboratory Press, Cold Spring Harbor, NY. Lentivirus Transduction of Hematopoietic Cells.

Sun, G., Li, H. and Rossi, J.J. (2007) In Methods in Enzymology, MicroRNA Methods, Vol. 427, Chapter 7, pp. 123-138, (Eds., J.J. Rossi and G.J. Hannon) Elsevier, Inc. San Diego, CA. Cloning and Detecting Signature MicroRNAs from Mammalian Cells.

Rossi, J.J. and Scherer, L.J. (2008) In RNA and the Regulation of Gene Expression, pp. 201-226, (Ed. Morris, K.V.) Caister Academic Press, Norfolk, NJ. Therapeutic Potential of RNA-mediated Control of Gene Expression: Options and Designs

Amarzguioui, M and Rossi, J.J. (2008) In *Methods in Molecular Biology*, Vol. 442, pp. 3-10, (Eds., S. Barik) Humana Press, Totowa, NJ. Principles of Dicer Substrate (D-siRNA) Design and function.

Morris, K.V. and Rossi, J.J. (2007) In Concepts in Genetic Medicine, pp. 207-222, (Eds. B. Dropulic and B. Carter) John Wiley & Sons, Inc., Hoboken, NJ. Lentiviral Vector Delivery of RNAi Triggers for the Treatment of HIV-1 Infection.

Li, M.-J. and Rossi J.J. (2008) In Methods in Molecular Biology, Gene Therapy Protocols, Vol. 1., Production and In Vivo Applications of Gene Transfer Vector, Vol. 1, pp. 287-299, (Ed. J.M. Le Doux) Humana Press Inc., Totowa, NJ. Lentiviral Vector Delivery of siRNA and shRNA Encoding Genes into Cultured and Primary Hematopoietic Cells.

Kim, D.-H., Behlke, M., and Rossi, J.J. (2008) In Methods in Molecular Biology 488, RNA-Protein Interaction Protocols, pp. 367-381, (Eds. R.-J. Lin) Humana Press Inc., Totowa, NJ. Designing and Utilization of siRNAs Targeting RNA Binding Proteins.

Scherer, L.J. and Rossi, J.J. (2009) In Gene and Cell Therapy: Therapeutic Mechanisms and Strategies, pp. 881-887, (Eds. N.S. Templeton) CRC Press Inc., Boca Raton, FL. RNA interference-based gene therapy strategies for the treatment of HIV infection.

Gaur, R.K. and Rossi, J.J. (2009) Regulation of Gene Expression by Small RNAs, (Eds. Gaur, R.K. and Rossi, J.J.) CRC Press Inc., Boca Raton, FL.

Kim, D. and Rossi, J.J. (2009) In Regulation of Gene Expression by Small RNAs, Chapter 20, p. 393-404, (Eds. Gaur, R.K. and Rossi, J.J.) CRC Press Inc., Boca Raton, FL., Mammalian Transcriptional Gene Silencing by Small RNAs.

Kim, D.H. and Rossi, J.J. (2008) In Methods in Molecular Biology, Therapeutic Applications of RNAi, (Eds. D.M. Rondinone and J. Reidhaar-Olson) The Humana Press Inc., Totowa, NJ. Transcriptional Gene Silencing using Small RNAs. (In Press)

Kim, D., and Rossi, J.J. (2008) In Current Protocols in Nucleic Acid Chemistry, (Eds. S. Beaucage, D.E. Bergstrom, P. Herdewijn and A. Matsuda) John Wiley & Sons, Inc., Hoboken, NJ. Overview of Gene Silencing by RNA Interference. (In Press)

Scherer, L., Weinberg, M. and Rossi, J.J. (2009) In *Therapeutic Oligonucleotides*, Chapter 14, pp. 316-328, (Eds., Kurreck, J.) Royal Society of Chemistry, Cambridge, United Kingdom, RNA Based Therapies for Treatment of HIV Infection. (In Press)